

The 2017 David Sackett Trial of the Year Award

The Systemic Therapy in Advancing or Metastatic Prostate Cancer: Evaluation of Drug Efficacy (STAMPEDE, NCT00268476) trial and Efficacy and Safety of Voretigene Neparvovec (AAV2-hRPE65v2) in Patients with RPE65-mediated Inherited Retinal Dystrophy (NCT00999609) are co-recipients of the prestigious David Sackett Trial of the Year Award, presented annually by the Society for Clinical Trials.

Matt Sydes, Ph.D., Senior Scientist, Senior Statistician and Deputy Director MRC CTU Hubs for Trials Methodology Research, will accept the award on behalf of the STAMPEDE trial team. Dr. Fan-fan Yu, Sc.D., Senior Statistical Scientist at Statistics Collaborative and Dr. Kathleen Z. Reape, M.D., head of Clinical Research and Development at Spark Therapeutics, Inc. will accept the award on behalf of the Voretigene Neparvovec trial team. The co-recipients will present their trials on May 23, 2018 at the Society's 39th Annual Meeting in Portland, Oregon.

Prostate cancer is the most common male cancer with more than one million men diagnosed each year. STAMPEDE is a trial for men who have a high-risk disease which has spread elsewhere (metastatic) or has other aggressive features. STAMPEDE has twice provided practice-changing data from randomised comparisons, once showing improved survival by adding docetaxel chemotherapy to the five-decade standard-of-care of long-term hormone therapy, and most recently improving survival by adding abiraterone to the standard-of-care. STAMPEDE is the first full implementation of the multi-arm multi-stage (MAMS) platform design. The design allows the contemporaneous evaluation of a number of treatments, comparing them to a common standard-of-care through phase II and phase III elements.

Nearly all patients who have inherited retinal dystrophy associated with biallelic *RPE65* mutations experience progressive retinal degeneration, vision loss, and progression to blindness. No treatment was available until December 19, 2017 when the US FDA approved voretigene neparvovec-rzyl for the treatment of these patients. Approval was based upon a Phase 3 trial that evaluated whether voretigene neparvovec-rzyl improved bilateral functional vision. In the absence of prior therapies and clinical trials in this rare disease, a novel functional vision endpoint tailored to the specific disease and its clinical deficits, the multi-luminance mobility test (MLMT) was developed and validated. Voretigene neparvovec-rzyl was superior to controls with respect to the change in bilateral MLMT score after one year. Improvements in visual function as measured by full-field light sensitivity threshold testing and visual field exams were also observed. Voretigene neparvovec-rzyl represents the first available treatment for these patients who would otherwise progress to blindness. As the first trial providing the basis for US regulatory approval for a gene-based therapy, it provides an important break-through in the development of gene-based therapies for inherited genetic diseases.

Each year since 2008, the SCT has been awarded to a randomized, controlled trial published (either electronically or in print) in the previous calendar year that best fulfills the following standards:

- It improves the lot of humankind.
- It provides the basis for a substantial, beneficial change in health care.
- It reflects expertise in subject matter, excellence in methodology, and concern for study participants.
- It overcomes obstacles in implementation.
- The presentation of its design, execution, and results is a model of clarity and intellectual soundness.

Nominations came from Society members, investigators, and interested scholars from around the world. The 2017 Trial of the Year selection committee included Marc Buyse, Henry “Chip” Chambers, Bob Dworkin, Susan Ellenberg, Scott Evans (Chair), Dean Follmann, Toshimitsu Hamasaki, Frank Rockhold, and Yves Rosenberg.

The 2018 Trial of the Year Selection Committee will issue a call for nominations in fall, 2018. Visit www.sctweb.org for updates.

About the Society for Clinical Trials: The Society for Clinical Trials, created in 1978, is an international professional organization dedicated to the development and dissemination of knowledge about the design, conduct, analyses, and reporting of government and industry-sponsored clinical trials and related health care research methodologies. Visit www.sctweb.org.

About the Trial of the Year: Find a list of past Trials of the Year on www.sctweb.org.